

2018 Current Fiscal Year Report: Sickle Cell Disease Advisory Committee

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1. Department or Agency

Department of Health and Human Services

2. Fiscal Year

2018

3. Committee or Subcommittee

Sickle Cell Disease Advisory Committee

3b. GSA Committee No.

1026

4. Is this New During Fiscal Year?

No

5. Current Charter

06/30/2017

6. Expected Renewal Date

06/30/2019

7. Expected Term Date

8a. Was Terminated During Fiscal Year?

No

8b. Specific Termination Authority

8c. Actual Term Date

9. Agency Recommendation for Next Fiscal Year

Continue

10a. Legislation Req to Terminate?

Not Applicable

10b. Legislation Pending?

Not Applicable

11. Establishment Authority Authorized by Law

12. Specific Establishment Authority

42 U.S.C. 282(b)(16)

13. Effective Date

11/04/1988

14. Committee Type

Continuing

14c. Presidential?

No

15. Description of Committee Scientific Technical Program Advisory Board

16a. Total Number of Reports

No Reports for this Fiscal Year

17a. Open 1 17b. Closed 0 17c. Partially Closed 0 Other Activities 0 17d. Total 1 Meetings and Dates

Purpose	Start	End
Scientific Technical Program Advisory Board	01/26/2018	- 01/26/2018

Number of Committee Meetings Listed: 1

	Current FY	Next FY
18a(1). Personnel Pmts to Non-Federal Members	\$2,000.00	\$2,000.00
18a(2). Personnel Pmts to Federal Members	\$0.00	\$0.00
18a(3). Personnel Pmts to Federal Staff	\$42,451.00	\$43,258.00
18a(4). Personnel Pmts to Non-Member Consultants	\$0.00	\$0.00
18b(1). Travel and Per Diem to Non-Federal Members	\$4,860.00	\$4,903.00
18b(2). Travel and Per Diem to Federal Members	\$0.00	\$0.00
18b(3). Travel and Per Diem to Federal Staff	\$0.00	\$0.00
18b(4). Travel and Per Diem to Non-member Consultants	\$0.00	\$0.00
18c. Other(rents,user charges, graphics, printing, mail, etc.)	\$0.00	\$0.00
18d. Total	\$49,311.00	\$50,161.00
19. Federal Staff Support Years (FTE)	0.30	0.30

20a. How does the Committee accomplish its purpose?

The Sickle Cell Disease Advisory Committee is composed of highly qualified individuals with expertise in the areas of basic and clinical research, translational science, genetics and community programs related to sickle cell disease, as well as members of the patient community with leadership in programs related to sickle cell disease and government agencies that have oversight responsibility for health care delivery to individuals with genetic disorders. The Committee makes recommendations on research priorities conducted or supported by the Institute. The Committee also makes recommendations and develops long range plans of basic, clinical, applied, health education, and prevention related research concerning the scientific aspects of research programs on the nature, causes, diagnosis, treatment, and prevention of Sickle Cell Disease. NHLBI in turn provides feedback and updates its response to action items generated at each meeting.

20b. How does the Committee balance its membership?

The Committee consists of 15 appointed members, five selected from authorities knowledgeable in clinical fields related to sickle cell disease, three members selected from experts in basic science fields related to sickle cell disease, three members selected from experts in translational science, and four public members selected with demonstrated leadership in programs related to sickle cell disease. The strength of the Committee is the diversity of its membership that represents disciplines from basic science to public health administration. It is further augmented by officials of Federal agencies involved in sickle cell services and related activities, e.g., Center for Disease Control and Prevention, and the Health Resources and Services Administration.

20c. How frequent and relevant are the Committee Meetings?

The Committee normally meets approximately two times per year. These meeting(s) include scientific presentations of ongoing or pending projects, program review of current and planned efforts, as well as open discussion regarding programmatic needs and direction. Members are asked to contribute expertise and specific efforts for future initiatives, conferences, and further advisory efforts that might prove helpful. One meeting was held in FY2018 on January, 26, 2018.

20d. Why can't the advice or information this committee provides be obtained elsewhere?

This Committee, with professional and public members, is knowledgeable about sickle cell disease and biology relevant to sickle cell pathogenesis and serves as an effective mechanism for bridging the gap between the Federal programs and the community, and for providing advice to the National Sickle Cell Program. It provides an important focus for

obtaining information from all Federal agencies that is not available through other mechanisms.

20e. Why is it necessary to close and/or partially closed committee meetings?

N/A

21. Remarks

Reports: This committee did not produce any public reports. Members: Derek A. Robertson was reappointed to the Committee for 2 additional years. Sonja L. Banks resigned from the committee December 31, 2017. The DFO and Committee Decision Maker positions are held by the same individual based on assigned duties within the Institute.

Designated Federal Officer

W Keith Hoots Director, Division of Blood Diseases and Resources

Committee Members	Start	End	Occupation	Member Designation
BANKS, SONJA	06/29/2013	12/31/2017	PRESIDENT AND CHIEF OPERATING OFFICER	Special Government Employee (SGE) Member
COLLINS, FRANCIS	08/17/2009	01/20/2021	DIRECTOR	Ex Officio Member
HIGH, KATHERINE	11/30/2014	06/30/2018	PRESIDENT AND CHIEF SCIENTIFIC OFFICER	Special Government Employee (SGE) Member
KANTER-WASHKO, JULIE	09/18/2016	06/30/2020	ASSISTANT PROFESSOR	Special Government Employee (SGE) Member
KLINGS, ELIZABETH	01/08/2017	06/30/2019	ASSOCIATE PROFESSOR OF MEDICINE	Special Government Employee (SGE) Member
MILLER, SHIRLEY	12/27/2016	06/30/2019	SICKLE CELL DISEASE PROGRAM MANAGER	Special Government Employee (SGE) Member
PACE, BETTY	12/27/2016	06/30/2019	PROFESSOR AND FRANCIS J. TEDESCO DISTINGUISHED CHAIR	Special Government Employee (SGE) Member
PANEPINTO, JULIE	08/21/2016	06/30/2019	PROFESSOR	Special Government Employee (SGE) Member
PORTEUS, MATTHEW	09/18/2016	06/30/2019	ASSOCIATE PROFESSOR	Special Government Employee (SGE) Member
ROBERTSON, DEREK	12/27/2016	06/30/2020	VICE PRESIDENT REGULATORY AFFAIRS	Special Government Employee (SGE) Member
VERCELLOTTI, GREGORY	01/08/2017	06/30/2019	PROFESSOR OF MEDICINE	Special Government Employee (SGE) Member
WALTERS, MARK	09/18/2016	06/30/2020	DIRECTOR	Special Government Employee (SGE) Member
WHITTEN-SHURNEY, WANDA	01/08/2017	06/30/2020	CHIEF EXECUTIVE OFFICER	Special Government Employee (SGE) Member
ZELTZER, LONNIE	01/08/2017	06/30/2019	PROFESSOR AND DIRECTOR OF THE PEDIATRIC PAIN PROGRAM	Special Government Employee (SGE) Member

Number of Committee Members Listed: 14

Narrative Description

The goal of NIH research is to acquire new knowledge to help prevent, detect, diagnose,

and treat disease and disability from the rarest genetic disorder to the common cold. The NIH mission is to uncover new knowledge that will lead to better health for everyone. NIH works toward that mission by the establishment of a Sickle Cell Disease Advisory Committee to provide advice and guidance concerning the Sickle Cell Disease Program, Division of Blood Diseases and Resources, National Heart, Lung, and Blood Institute.

What are the most significant program outcomes associated with this committee?

Checked if Applies

- | | |
|---|-------------------------------------|
| Improvements to health or safety | <input checked="" type="checkbox"/> |
| Trust in government | <input type="checkbox"/> |
| Major policy changes | <input type="checkbox"/> |
| Advance in scientific research | <input type="checkbox"/> |
| Effective grant making | <input type="checkbox"/> |
| Improved service delivery | <input type="checkbox"/> |
| Increased customer satisfaction | <input type="checkbox"/> |
| Implementation of laws or regulatory requirements | <input type="checkbox"/> |
| Other | <input type="checkbox"/> |

Outcome Comments

N/A

What are the cost savings associated with this committee?

Checked if Applies

- | | |
|----------------------------|-------------------------------------|
| None | <input checked="" type="checkbox"/> |
| Unable to Determine | <input type="checkbox"/> |
| Under \$100,000 | <input type="checkbox"/> |
| \$100,000 - \$500,000 | <input type="checkbox"/> |
| \$500,001 - \$1,000,000 | <input type="checkbox"/> |
| \$1,000,001 - \$5,000,000 | <input type="checkbox"/> |
| \$5,000,001 - \$10,000,000 | <input type="checkbox"/> |
| Over \$10,000,000 | <input type="checkbox"/> |
| Cost Savings Other | <input type="checkbox"/> |

Cost Savings Comments

NIH supported basic and clinical research accomplishments often take many years to unfold into new diagnostic tests and new ways to treat and prevent diseases.

What is the approximate Number of recommendations produced by this committee for the life of the committee?

Number of Recommendations Comments

The SCDAC meeting on January 26, 2018 was devoted to the topic of The NIH Cure Sickle Cell Disease Initiative as well as an overview of the entire NHLBI sickle cell disease program. The presentations included a report on the Cure SCD Initiative and an update of the stem cell transplant protocols for adults with sickle cell disease. Katherine High, MD gave a presentation on her experience in the development of gene therapy for a rare eye disease, and Matthew Porteus, MD, PhD gave a talk on the scientific basis of gene editing. After these presentations, there was a wide-ranging discussion on NHLBI's future strategies to pursue first-in-human clinical vectors for potentially curing sickle cell disease. The Advisory Committee wholeheartedly endorsed the current programmatic goals that the NHLBI has proposed.

What is the approximate Percentage of these recommendations that have been or will be Fully implemented by the agency?

0%

% of Recommendations Fully Implemented Comments

Due to the complexity of the recommendations made by this committee, staff is unable to determine which recommendations have been fully or partially implemented solely in response to this committee's activities.

What is the approximate Percentage of these recommendations that have been or will be Partially implemented by the agency?

0%

% of Recommendations Partially Implemented Comments

Due to the complexity of the recommendations made by this committee, staff is unable to determine which recommendations have been fully or partially implemented solely in response to this committee's activities.

Does the agency provide the committee with feedback regarding actions taken to implement recommendations or advice offered?

Yes ☒ No ☐ Not Applicable ☐

Agency Feedback Comments

At each meeting, the Executive Secretary provides status updates on previous recommendations made by the committee.

What other actions has the agency taken as a result of the committee's advice or recommendation?

Checked if Applies

- | | |
|-----------------------------------|--------------------------|
| Reorganized Priorities | <input type="checkbox"/> |
| Reallocated resources | <input type="checkbox"/> |
| Issued new regulation | <input type="checkbox"/> |
| Proposed legislation | <input type="checkbox"/> |
| Approved grants or other payments | <input type="checkbox"/> |
| Other | <input type="checkbox"/> |

Action Comments

N/A

Is the Committee engaged in the review of applications for grants?

No

Grant Review Comments

N/A

How is access provided to the information for the Committee's documentation?

Checked if Applies

- | | |
|---------------------------|-------------------------------------|
| Contact DFO | <input checked="" type="checkbox"/> |
| Online Agency Web Site | <input checked="" type="checkbox"/> |
| Online Committee Web Site | <input checked="" type="checkbox"/> |
| Online GSA FACA Web Site | <input checked="" type="checkbox"/> |
| Publications | <input type="checkbox"/> |
| Other | <input type="checkbox"/> |

Access Comments

N/A